Stem Cell Agency Board Approves Two Early-Stage Research Programs Targeting Cartilage Damage

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Oakland, CA – Every year millions of Americans suffer damage to their cartilage, either in their knee or other joints, that can eventually lead to osteoarthritis, pain and immobility. Today the governing Board of the California Institute for Regenerative Medicine (CIRM) approved two projects targeting repair of damaged cartilage.

The projects were among 17 approved by CIRM as part of the DISC2 Quest Discovery Program. The program promotes the discovery of promising new stem cell-based and gene therapy technologies that could be translated to enable broad use and ultimately, improve patient care.

Dr. Darryl D'Lima and his team at Scripps Health were awarded $1,620,645 to find a way to repair a torn meniscus. Every year around 750,000 Americans experience a tear in their meniscus, the cartilage cushion that prevents the bones in the knee grinding against each other. These injuries accelerate the early development of osteoarthritis, for which there is no effective treatment other than total joint replacement, which is a major operation. There are significant socioeconomic benefits to preventing disabling osteoarthritis. The reductions in healthcare costs are also likely to be significant.

The team will use stem cells to produce meniscal cells in the lab. Those are then seeded onto a scaffold made from collagen fibers to create tissue that resembles the knee meniscus. The goal is to show that, when placed in the knee joint, this can help regenerate and repair the damaged tissue.

This research is based on an earlier project that CIRM funded. It highlights our commitment to helping good science progress, hopefully from the bench to the bedside where it can help patients.

Dr. Kevin Stone and his team at The Stone Research Foundation for Sports Medicine and Arthritis were awarded $1,316,215 to develop an approach to treat and repair damaged cartilage using a patient's own stem cells.

They are using a paste combining the patient's own articular tissue as well as Mesenchymal Stem Cells (MSC) from their bone marrow. This mixture is combined with an adhesive hydrogel to form a graft that is designed to support cartilage growth and can also stick to surfaces without the need for glue. This paste will be used to augment the use of a microfracture technique, where micro-drilling of the bone underneath the cartilage tear brings MSCs and other cells to the fracture site. The hope is this two-pronged approach will produce an effective and functional stem cell-based cartilage repair procedure.

If effective this could produce a minimally invasive, low cost, one-step solution to help people with cartilage injuries and arthritis

The full list of successful DISC2 applicants is below.

The CIRM Board also approved a new training program called COMPASS (Creating Opportunities through Mentorship and Partnership Across Stem Cell Science). The program will fill a critical need for skilled research practitioners who understand and contribute at all levels in the translation of science to medicine, from bench to bedside.

The objective of the COMPASS Training Program is to prepare a diverse group of undergraduate students for careers in regenerative medicine through the creation of novel recruitment and support mechanisms that identify and foster untapped talent within populations that are historically under-represented in the biomedical sciences. It will combine hands-on research with mentorship experiences to enhance transition of students to successful careers. A parallel objective is to foster greater awareness and appreciation of diversity, equity and inclusion in trainees, mentors, and other program participants

The CIRM Board approved investing $58.22 million for up to 20 applications for a five-year duration.

"This new program highlights our growing commitment to creating a diverse workforce, one that taps into communities that have been historically under-represented in the biomedical sciences," says Dr. Maria T. Millan, President and CEO of CIRM. "The COVID19 pandemic made it clear that the benefits of scientific discovery are not always accessible to communities that most need them. CIRM is committed

to tackling these challenges by creating a diverse and dedicated workforce that can meet the technical demands of taking novel treatment ideas and making them a reality."

The Board also approved a new $80 million concept plan to expand the CIRM Alpha Stem Cell Clinic Network. The Network clinics are all in top California medical centers that have the experience and the expertise to deliver high-quality FDA-authorized stem cell clinical trials to patients.

There are currently five Alpha Clinics – UC San Diego; UCLA/UC Irvine; City of Hope; UCSF; UC Davis – and since 2015 they have hosted more than 105 clinical trials, enrolled more than 750 patients in these trials, and generated more than $95 million in industry contracts.

Each award will provide up to $8 million in funding over a five-year period. The clinics will have to include:

- A demonstrated ability to offer stem cell and gene therapies to patients as part of a clinical trial.
- Programs to help support the career development of doctors, nurses, researchers or other medical professionals essential for regenerative medicine clinical trials.
- A commitment to data sharing and meeting CIRM's requirements addressing issues of diversity, equity and inclusion and meeting the needs of California's diverse patient population

Application	**Title**	**Principal Investigator and Institution**	**Amount**
DISC2-13212	*Preclinical development of an exhaustion-resistant CAR-T stem cell for cancer immunotherapy*	**Ansuman Satpathy - Stanford University**	$ 1,420,200
DISC2-13051	*Generating deeper and more durable BCMA CAR T cell responses in Multiple Myeloma through non-viral knockin/knockout multiplexed genome engineering*	Julia Carnevale – UC San Francisco	$ 1,463,368
DISC2-13020	*Injectable, autologous iPSC-based therapy for spinal cord injury*	**Sarah Heilshorn -** **Stanford University**	$789,000
DISC2-13009	*New noncoding RNA chemical entity for heart failure with preserved ejection fraction.*	Eduardo Marban – Cedars-Sinai Medical Center	$1,397,412
DISC2-13232	*Modulation of oral epithelium stem cells by RSpo1 for the prevention and treatment of oral mucositis*	**Jeffrey Linhardt -** Intact Therapeutics Inc.	$942,050

DISC2-13077	*Transplantation of genetically corrected iPSC-microglia for the treatment of Sanfilippo Syndrome (MPSIIIA)*	Mathew Blurton-Jones – UC Irvine	$1,199,922
DISC2-13201	*Matrix Assisted Cell Transplantation of Promyogenic Fibroadipogenic Progenitor (FAP) Stem Cells*	Brian Feeley – UC San Francisco	$1,179,478
DISC2-13063	*Improving the efficacy and tolerability of clinically validated remyelination-inducing molecules using developable combinations of approved drugs*	**Luke Lairson -** Scripps Research Inst.	$1,554,126
DISC2-13213	*Extending Immune-Evasive Human Islet-Like Organoids (HILOs) Survival and Function as a Cure for T1D*	**Ronald Evans – The Salk Institute for Biological Studies**	$1,523,285
DISC2-13136	*Meniscal Repair and Regeneration*	**Darryl D'Lima – Scripps Health**	$1,620,645
DISC2-13072	*Providing a cure for sphingosine phosphate lyase insufficiency syndrome (SPLIS) through adeno-associated viral mediated SGPL1 gene therapy*	Julie Saba – UC San Francisco	$1,463,400
DISC2-13205	*iPSC-derived smooth muscle cell progenitor conditioned medium for treatment of pelvic organ prolapse*	Bertha Chen – Stanford University	$1,420,200
DISC2-13102	*RNA-directed therapy for Huntington's disease*	**Gene Wei-Ming Yeo** - UC San Diego	$1,408,923

DISC2-13131	*A Novel Therapy for Articular Cartilage Autologous Cellular Repair by Paste Grafting*	Kevin Stone - The Stone Research Foundation for Sports Medicine and Arthritis	$1,316,215
DISC2-13013	*Optimization of a gene therapy for inherited erythromelalgia in iPSC-derived neurons*	**Ana Moreno - Navega Therapeutics**	$1,157,313
DISC2-13221	*Development of a novel stem-cell based carrier for intravenous delivery of oncolytic viruses*	**Edward Filardo - Cytonus Therapeutics, Inc.**	$899,342
DISC2-13163	*iPSC Extracellular Vesicles for Diabetes Therapy*	Song Li – UC Los Angeles	$1,354,928

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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